Press Release

AMO Pharma Announces Collaboration with Population Health Research Institute to Advance Proof of Concept Clinical Trial to Assess Efficacy of Tideglusib in Treatment of Arrhythmogenic Cardiomyopathy

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LONDON, Feb. 15, 2024 /PRNewswire/ -- AMO Pharma Limited ("AMO Pharma"), a privately held clinical-stage specialty biopharmaceutical company focusing on rare genetic disorders with limited or no treatment options, today announced a collaboration with Population Health Research Institute (PHRI), a joint institute of McMaster University and Hamilton Health Sciences in Canada, to advance a clinical proof of concept trial to assess the efficacy of AMO-02 (tideglusib), AMO Pharma's investigational oral glycogen synthase kinase 3 beta (GSK3β) inhibitor, in the treatment of genotype positive arrhythmogenic cardiomyopathy (ACM).

The study, entitled "Targeted Therapy with Glycogen Synthase Kinase-3 Inhibition for Arrhythmogenic Cardiomyopathy" (TaRGET), will be led by the research team at PHRI and will involve collaboration with multiple organizations in addition to AMO Pharma including the Hearts in Rhythm Organization (HiRO). Enrollment is set to begin in mid-2024 at 20 sites across Canada and will include a total of 120 participants who will be randomized in a 1:1 ratio.

"It can be a helpless feeling to carry a gene for ACM and know that, no matter how you live your life, you may someday develop a deadly form of heart disease," said Jason Roberts, scientist at PHRI and TaRGET study principal investigator, adding, "Encouraged by earlier studies in animal models, we are hopeful that tideglusib may serve as an effective treatment that can dramatically improve the health of people living with ACM. The TaRGET trial is a critical first step in evaluating this potential."

ACM is a form of genetic heart disease characterized by scarring in the heart muscle. This scarring increases the risk of dangerously fast heart rhythms that can lead to sudden cardiac death (SCD). There are currently no approved medical therapies that have been shown to prevent progression of ACM. An implantable cardioverter defibrillator (ICD) is recommended for prevention of SCD for ACM patients considered at high risk for SCD.

In prior research, a high-throughput screen of bioactive compounds against a zebrafish model of ACM identified a small molecule classified as a GSK3β inhibitor that successfully prevented and rescued the phenotype, findings that have subsequently been reproduced in a series of ACM murine models. GSK3β is an enzyme that modulates the activity of a broad spectrum of intracellular signaling pathways, including the canonical Wnt/β-catenin pathway whose suppression has been suggested to exert an important role in ACM pathogenesis.

"As we continue to advance our research to assess the potential benefits of AMO-02 in the treatment of myotonic dystrophy, we are very pleased to be supporting this landmark research effort in ACM with the outstanding team at PHRI," said Mike Snape, chief scientific officer at AMO Pharma. "Based on extensive research that highlights the proven MOA of AMO-02 as a clinically safe and well tolerated GSK3β inhibitor, we look forward to progress in advancing this promising program that can bring new hope to people living with ACM and their families in the years ahead. This new data has the potential to build on the cardiac efficacy data previously reported in studies conducted in Duchenne muscular dystrophy by Professor Val Fajardo's team at Brock University."

About AMO Pharma
AMO Pharma is a clinical-stage specialty biopharmaceutical company working to identify and advance promising therapies for the treatment of serious and debilitating diseases in patient populations with significant areas of unmet need, including rare and severe childhood onset neurogenetic disorders with limited or no treatment options. In addition to developing AMO-02 for myotonic dystrophy, the company is also progressing AMO-01 as a clinical stage treatment for Phelan-McDermid syndrome and AMO-04 as a clinic-ready potential medicine for Rett syndrome and related disorders. AMO-02, AMO-01 and AMO-04 are investigational medicines that have not yet been approved for the treatment of patients anywhere in the world. Advice provided to AMO Pharma by regulators is under the condition that any scientific advice given is not legally binding with regards to any future application for the product concerned, neither on the part of MHRA/Commission on Human Medicines (CHM) nor on the Company. Furthermore, advice cannot be taken as indicative of any future agreed position.

For more information, please visit the AMO Pharma website at http://www.amo-pharma.com/.

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